Patient Perspective on BCMA-**Targeted Therapies for Multiple** Myeloma: A Survey Conducted in a Patient Advocacy Group

BACKGROUND

- Multiple myeloma (MM) is a cancer of the plasma cells and is the second most prevalent hematologic cancer, with an estimation of 35,730 new cases in 2023 in the United States¹ - Approximately 41.5% of patients with MM die of the disease annually and the five-year
- survival rate is approximately 51%²
- Over the past 15 years, patient survival has improved due to the introduction of innovative targeted therapies such as immunomodulatory drugs (IMiDs), proteasome inhibitors (PIs), monoclonal antibodies, and novel immunotherapies
- The emergence of B-cell maturation antigen (BCMA)-targeted therapies has improved clinical outcomes in patients with heavily pretreated disease. Current BCMA-targeted therapies that have received FDA approval for the treatment of relapsed/refractory multiple myeloma (RRMM) include chimeric antigen receptor T-cell (CAR T-cell) therapies, idecabtagene vicleucel (ide-cel) and ciltacabtagene autoleucel (cilta-cel), and anti-CD3/ anti-BCMA bispecific antibody, teclistamab³
- Little is known about patients' knowledge, decision-making processes, and openness to these novel BCMA-targeted therapies

OBJECTIVE

• The objective of this study is to gain insights into MM patients' perspectives on BCMA-targeted therapies. By understanding patient preferences, values, knowledge gaps, and willingness to try new therapies, healthcare providers can better support patients in making informed treatment decisions

METHODS

Patient Population

• All patients (>11,000) on the HealthTree Cure Hub platform, an online portal to help patients with plasma cell dyscrasias navigate their diseases, were notified of the survey via e-mail. Adult patients diagnosed with MM who consented to the study and filled out the online survey were included in the analysis

Study Design

• This is a cross-sectional survey completed by patients with MM enrolled in HealthTree Cure Hub. An 18-question online survey developed by the research team was reviewed and adjusted by the HealthTree Patient Advocacy Panel and a physician panel. Patient consent was obtained online through the HealthTree Cure Hub portal prior to the survey. The online survey took place between October 28, 2022, and January 12, 2023. Deidentified responses were analyzed descriptively and reported in aggregate. Statistical tests were done to understand whether responses would differ by patients' MM status and prior therapy use

Survey Instrument

• The 18-question patient online survey instrument included questions assessing patients' perspectives in three general domains, including treatment decisions process, expectation and evaluation of MM treatment, and their perspectives and information needs related to BCMA-targeted therapies including CAR T-cell therapy and bispecific antibodies. Content of the questionnaire is outlined in **Table 1**. Respondents were not required to answer all questions in the questionnaire. For some questions, respondents were asked to rank items listed for each question. They were not required to rank all items. There were also certain questions where respondents were asked to select all items that applied

Data Analysis

- All variables were analyzed descriptively for all respondents
- Rank score was calculated as the sum of the inverse rank order by the respondent, then summed across the sample order data and reported from the highest to the lowest
- Responses were analyzed for all respondents and then stratified by patients' MM status (newly diagnosed multiple myeloma [NDMM] vs. RRMM) and by the number of prior lines of therapy (LOT) (NDMM vs. 1-3 prior LOTs vs. 4+ prior LOTs)

TABLE 1. Online Multiple Myeloma Patient Survey Questionnaire Content

Treatment decisions process

People involved in treatment decision-making, including healthcare providers (HCPs) and non-HCPs

Patients' level of involvement in treatment decision-making

Patients' mode of transportation and distance from treatment center

Patients' expectation and evaluation of MM treatment

Factors that encourage one to consider changing treatment

Trade-offs that the patient would be willing to make

Side effects that would make a patient choose not to receive MM treatment

Challenges that the patient is facing regarding MM treatment

Openness to new therapies

Factors that are important when choosing MM treatment

Confidence level on other treatment options for MM if one relapses while on current treatment Evaluation of supporting materials/programs to support MM treatment experience

Patients' perspectives and information needs related to BCMA-targeted therapies for MM

Awareness of BCMA-targeted therapy and sources

Likelihood to try a CAR T-cell therapy if available

Likelihood to try a bispecific antibody if available

Needs of additional information on CAR T-cell therapy to support patient decision-making Needs of additional information on bispecific antibodies to support patient decision-making BCMA, B-cell maturation antigen; CAR-T, chimeric antigen receptor T cell; HCP, healthcare provider; MM, multiple myeloma.

Presented at the European Hematology Association (EHA) Congress; June 8-11, 2023; Frankfurt, Germany

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RESULTS

• 325 patients with MM participated in the survey. Among 218 patients who had complete clinical records in the database, 71 (33%) had NDMM and 86 (39%) had 1-3 prior LOTs Among the 61 (28%) patients who had at least 4 prior LOTs, 90% were triple-class exposed (defined as having received a proteasome inhibitor, an immunomodulatory drug, and an anti-CD38 monoclonal antibody). The demographic characteristics, disease status, and treatment history for the overall population are shown in **Table 2**

TABLE 2. Demographics, Disease Status, and Treatment History of Patients With Multiple Myeloma Who Participated in the Survey

Demographics (N = 325)	
Age (319 respondents, 98% of total surveyed)	
Age (mean ± SD)	66 ± 8 years
N (% of total respondents)	
Sex (319 respondents, 98% of total surveyed)	
Female	171 (54)
Ethnicity (245 respondents, 75% of total surveyed)	
Hispanic or Latino	37 (15)
Racial Background (253 respondents, 78% of total surveyed)	
White (original ancestry from Europe, Middle East, North Africa)	228 (90)
Black or African American (original ancestry from Africa)	15 (6)
Other	9 (4)
Type of Insurance (246 respondents,76% of total surveyed) Respondents asked to select all that apply	
Private commercial insurance through work	109 (44)
Private commercial Insurance through personal plan	20 (8)
Medicare Part B	88 (36)
Medicare Part A	87 (35)
Medicare Part D	57 (23)
Medigap or Medicare supplemental	47 (19)
Medicare Advantage	34 (14)
Medicaid	5 (2)
Other	20 (8)
Highest Level of Education (254 respondents, 78% of total surveyed)	
College and above	192 (76)
Some college or associate degree	36 (14)
High school or below	25 (10)
Other	1 (< 1)
Participant Disease Status	
R-ISS Stage at Diagnosis (176 respondents, 54% of total surveyed)	
Stage I	50 (28)
Stage II	65 (37)
Stage III	42 (24)
I don't know	19 (11)
Treatment History	
Current or Most Recent Line of Therapy (218 respondents, 67% of total surveyed	d)
LOT (mean ± SD)	3.05 ± 2.64
1	71 (33)
2	59 (27)
3	27 (12)
4+ (4L-16L)	61 (28)

4+ (4L-16L)

LOT, line of therapy; MM, multiple myeloma; R-ISS, Revised Multiple Myeloma International Staging System.

- Of the 290 MM patients who responded to the question about their openness to trying a new therapy, the majority (95%) reported being open; 26% reported being open to trying right away, while 43% reported being open but wanting more information on safety and efficacy (Table 3) • Respondents reported a high level of awareness for the BCMA-targeted therapy for MM, including CAR T-cell therapy and bispecific antibodies, with 92% reporting having heard of
- BCMA-targeted therapies (**Table 3**) • 65% and 74% of respondents to questions on likelihood to try a CAR T-cell therapy and bispecific antibody, respectively, reported a high level of likelihood (likely or very likely) to try these therapies. In addition, 16% and 13% of respondents to these two survey questions (CAR T-cell therapy and bispecific antibody, respectively) indicated they needed more information to decide (Table 3)
- When asked about information needed to support decision-making for CAR T-cell or bispecific therapies, the most requested domains of information reported by respondents for both therapies were efficacy, side effects (SEs), eligibility, and administration process. "How soon can I receive it?" was ranked higher for bispecific therapy, while "Where can I receive it?" was ranked higher for CAR T-cell therapy, relatively (Table 3)
- When asked about MM therapy preference, assuming the same efficacy and duration of response, 69% of respondents reported preference for therapy with lower risk of severe SEs but requiring continuous dosing with no treatment-free interval, as opposed to the therapy that is given once followed by a treatment-free interval but with a potentially higher risk of severe SEs (31%) (**Table 3**)

Including BCMA-Targeted Therapies	
Total No. of MM Patients Surveyed = 325	n (% of total respondents)
Openness to New Therapies Respondents (290, 89% of total surveyed)	
Very open, if eligible, I want to try as soon as possible	76 (26)
Open, but would like to wait for more data on efficacy and safety	125 (43)
Open, if other patients I know have tried it	5 (2)
Open, if my health care provider recommends it	70 (24)
I'm not interested in trying new therapy at the moment	12 (4)
Not sure	2 (1)
Awareness of BCMA-Targeted Therapy and Sources ^a	
Respondents (216, 67% of total surveyed)	
Yes, from online search	120 (56)
Yes, from my healthcare providers	79 (37)
Yes, from other resources	67 (31)
Yes, from a clinical trial I participated in	21 (10)
Yes, from family and friends	12 (6)
Yes, from media advertisement	10 (5)
No	17 (8)
Likelihood of Trying a CAR T-Cell Therapy If Available	
Respondents (198, 61% of total surveyed)	
I have already received one	16 (8)
Very likely or Likely	119 (60)
Neutral	21 (11)
Very Unlikely or Unlikely	11 (6)
I need more information to decide	31 (16)
Likelihood of Trying a Bispecific Antibody If Available Respondents (198, 61% of total surveyed)	
Very likely or Likely	146 (74)
Neutral	22 (11)
Unlikely	3 (2)
I need more information to decide	26 (13)
I have not heard of a bispecific antibody	1 (1)
Additional Information on CAR T-Cell Therapy Needed to Support Your Decision MM Treatment ^b Respondents (95, 29% of total surveyed)	for Your
Efficacy – how well the therapy will provide me the desired clinical outcome	83 (87)
Side effects	68 (72)
Am I the right patient to receive it	61 (64)
What is the administration process and procedure	51 (54)
Costs to me	53 (56)
Where I can receive it	50 (53)
How will this therapy impact my family or caregivers	42 (44)
How often I need to receive it	45 (47)
How soon I can receive it	-3 (-7) 19 (52)
Additional Information on Bispecific Antibodies Needed to Support Your Decision	on for Your
Respondents (76, 23% of total surveyed)	
Efficacy – how well the therapy will provide me the desired clinical outcome	68 (89)
Side effects	56 (74)
Am I the right patient to receive it	48 (63)
What is the administration process and procedure	41 (54)
Costs to me	41 (54)
How soon I can receive it	37 (49)
How often I need to receive it	38 (50)
Where I can receive it	38 (50)
How will this therapy impact my family or caregivers	31 (41)
Assuming the Same Efficacy and Same Duration of Response, Which Therapy W	ould You
Choose for Your MM Respondents (292, 90% of total surveyed)	
"A therapy with less risk but requiring continuous dosing no troatmost free interval"	201(60)
"A therapy that is given once followed by a treatment-free interval but with a	
potentially higher risk of severe side effects"	91 (31)
всма, в-сен maturation antigen; CAR T-cell, chimeric antigen receptor T cell; MM, multiple myeloma.	

TABLE 3. Survey Results of Patients' Perspectives on New Therapies for Multiple Myeloma

^aRespondents were asked to "select all that apply." ^bRespondents were asked to "rank from the most to least important, ranking all is not required."

• To receive a treatment that brings improved outcomes, respondents indicated willingness to accept certain "trade-offs." The top acceptable trade-offs included frequent monitoring of SEs (61%) and initiating a new drug in a hospital setting (59%). Respondents were least willing to compromise on caregiver burden (22%) (**Figure 1**)

- The most acceptable SEs reported by respondents were those that were asymptomatic but would need routine monitoring to prevent serious complications (64%) and those that were cosmetic but non–life threatening (52%). SEs that were rare but could cause serious problems or were life-threatening were least acceptable (27%) to the respondents (**Figure 2**)
- Findings were consistent across subgroups of patients with NDMM, RRMM and different numbers of prior LOTs, with no statistically significant differences detected

FIGURE 1. Types of trade-offs respondents (298, 92% of total surveyed) were willing to make to receive multiple myeloma treatment that brings improved outcomes (% of total respondents)



FIGURE 2. Type of side effects that would make respondents (298, 92% of total surveyed) NOT want to receive a beneficial multiple myeloma treatment



CONCLUSIONS

- This study found a high level of openness in patients with MM to try BCMA-targeted CAR T-cell and bispecific therapies, if offered
- Information on efficacy, safety, availability, and eligibility may assist patients with their decision-making
- To receive a treatment that could be clinically beneficial, patients were willing to accept certain trade-offs, including regular AE monitoring and initiating a therapy in a hospital setting, as well as certain AEs
- Incorporating patients' goals, values, and preferences alongside clinical factors and other considerations may further optimize treatment decisions and improve patient outcomes

KEY TAKEAWAYS

- This is the first known study of patients' perspectives on the use of novel BCMA-targeted therapies for MM. It also evaluated the type of information patients considered necessary when deciding whether to utilize these therapies
- There is a high level of openness among patients with MM to receive BCMA-targeted therapies and acceptance of certain trade-offs when receiving clinically beneficial treatment • This study points to the importance of providing information related to treatment efficacy, safety, availability, and eligibility for patients' treatment decision-making

STRENGTHS

- This is the first known study on the perspectives of patients with MM toward the use of novel BCMA-targeted therapies and the type of information that is needed to assist patients in their decision-making process
- Patients' demographics, clinical characteristics, and treatment history have been collected regularly by HealthTree Cure Hub as part of their data dictionary. Availability of such data enables rapid survey administration, data collection, and analysis, and the richness of the data also allows subgroup analysis to be carried out to answer various research questions
- This study has a relatively large sample size for this rare-disease population. A targeted recruitment of minority populations on a nonprofit patient education, empowerment, and engagement platform likely played a key role in reaching a diverse group of patients to ensure that their perspectives were represented in the study

LIMITATIONS

- As a self-reported survey of patient perspectives on MM treatment, this study has the potential for bias, such as response, social desirability, and recall biases that are common in such a study design
- Patients who participated in the survey were likely representative of the entire MM population across several important domains, as was shown in prior research by HealthTree Foundation (in partnership with the Mayo Clinic). Of note, this research showed higher self-reported levels of disease and treatment knowledge, which may be indicative of more informed cancer patients. Thus, the result of the current study may reflect the choices and decisions of welleducated MM patients, which may not be representative of the overall MM population
- Patients participating in the survey were not required to respond to all questions or all the items in a question, resulting in incomplete data. Furthermore, for questions for which the respondents were asked to rank items, respondents were not required to rank everything, likely resulting in high heterogeneity of reporting results in those cases
- This study did not use any validated patient-reported outcome instruments. However, efforts were made to ensure the validity and reliability of the questionnaire through review by the HealthTree Patient Advocacy Panel and a physician panel, despite the absence of a test-retest analysis or convergent comparisons

REFERENCES

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